

APPENDIX

This appendix provides supplementary content not featured in the main paper, including a set of appendix tables. The appendix tables provide a list of groups of *CFTR* mutations (Exhibit A1), the disaggregated results for all-cause admissions for the initial label and expanded label subcohorts (Exhibit A2), and a replication of the analyses reported in the paper that were conducted using MarketScan Medicaid data (Exhibit A3).

List of Appendix Exhibits

1. Appendix Exhibit A1: Timeline of Food and Drug Administration approval of ivacaftor and label extensions
2. Appendix Exhibit A2: Subcohort analysis of inpatient admissions before and after initiating ivacaftor
3. Appendix Exhibit A3: Medicaid analysis of inpatient admissions among patients initiating ivacaftor

Appendix Exhibit A1: Timeline of Food and Drug Administration approval of ivacaftor and label extensions

Date of action	Approved CFTR mutations added	Age group
January 31, 2012	<i>G551D</i>	≥6 years
February 21, 2014	<i>G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R</i>	≥6 years
December 29, 2014	<i>R117H</i>	≥6 years
March 18, 2015	All indicated mutations	≥2 years
May 17, 2017	5 splicing mutations	≥2 years
August 1, 2017	23 residual function mutations	≥2 years

Appendix Exhibit A2: Subcohort analysis of inpatient admissions before and after initiating ivacaftor

		Before Ivacaftor				After Ivacaftor				Before-After Change	
Age (years)	N	1 or More Admits	% with Admit	# of Admits	Rate per person-year	1 or More Admits	% with Admit	# of Admits	Rate per person-year	% with Admit	Rate
Initial label subcohort: Patients who initiated ivacaftor, February 6, 2012 – February 21, 2014											
6-64	86	27	31%	45	0.52	11	13%	23	0.27	59%	49%
6-17	38	13	34%	24	0.63	4	11%	9	0.24	69%	63%
18-64	48	14	29%	21	0.44	7	15%	14	0.29	50%	33%
Expanded label subcohort: Patients who initiated ivacaftor, February 22, 2014 – December 31, 2015											
6-64	57	21	37%	37	0.65	9	16%	14	0.25	57%	62%
6-17	15	7	47%	8	0.53	4	27%	4	0.27	43%	50%
18-64	42	14	33%	29	0.69	5	12%	10	0.24	64%	66%

Source: Authors' analysis of MarketScan Commercial Claims data (Truven Health).

Notes: Patients had at least one filled ivacaftor prescription during February 2012-December 2015 and were continuously enrolled in employer-sponsored health plans for at least 12 months preceding and following that prescription. Age was based on the age in years at the time of the first filled prescription. Inpatient admissions (admits) during the 12 months before and after the first ivacaftor prescription were calculated for all causes. The initial label subcohort began ivacaftor until the initial FDA label with eligibility restricted to patients with a G551D mutation on the *CFTR* gene, i.e., by February 21, 2014. The extended label subcohort initiated treatment after the FDA extended the label on February 21, 2014 to include additional mutations (see Appendix Exhibit 1).

Appendix Exhibit A3: Medicaid analysis of inpatient admissions among patients initiating ivacaftor

			Before Ivacaftor				After Ivacaftor				Before-After Change	
All-cause admissions	Age (years)	N	1 or More Admits	% with Admit	Number of Admits	Rate (person/year)	1 or More Admits	% with Admit	Number of Admits	Rate (person/year)	% with Admit	Rate
	6-64	50	15	30%	26	0.52	10	20%	16	0.32	33%	38%
	6-17	43	11	26%	15	0.35	7	16%	10	0.23	36%	33%
	18-64	7	4	57%	11	1.57	3	43%	6	0.86	25%	45%
CF-related admissions	Age (years)	N	1 or more Admits	% with Admit	Number of Admits	Rate person/year	1 or more Admits	% with Admit	Number of Admits	Rate (person/year)	% with Admit	Rate
	6-64	50	3	6%	13	0.26	2	4%	7	0.14	33%	46%
	6-17	43	2	5%	10	0.23	2	5%	6	0.14	0%	40%
	18-64	7	1	14%	3	0.43	0	0%	1	0.14	100%	67%

Source: Authors' analysis of MarketScan Multi-State Medicaid Claims data (Truven Health).

Notes: Patients had at least one filled ivacaftor prescription during February 2012-December 2015 and were continuously enrolled in employer-sponsored health plans for at least 12 months preceding and following that prescription. Age was based on the age in years at the time of the first filled prescription. Inpatient admissions (admits) during the 12 months before and after the first ivacaftor prescription were calculated for all causes and those in which a diagnosis code for cystic fibrosis (CF) was recorded (CF-related).